

CORRECTED VERSION

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
14 July 2005 (14.07.2005)

PCT

(10) International Publication Number
WO 2005/063007 A1

(51) International Patent Classification⁷: **A01K 67/027 //**
C12N 9/14

(21) International Application Number:
PCT/NO2004/000397

(22) International Filing Date:
22 December 2004 (22.12.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/533,740 30 December 2003 (30.12.2003) US

(71) Applicant (for all designated States except US):
ULLEVÅL UNIVERSITETSSYKEHUS HF [NO/NO];
N-0407 Oslo (NO).

(72) Inventors; and

(75) Inventors/Applicants (for US only): **CHRISTENSEN,**
Geir [NO/NO]; Borgenveien 22A, N-0379 Oslo (NO).
ANDERSSON, Kristin Brevik [NO/NO]; Nyveien 15C,
N-1482 Nittedal (NO).

(74) Agent: **NILSEN, Esben, A.;** Zacco Norway AS, P.O.
Box 765, Sentrum, N-0106 Oslo (NO).

(81) Designated States (unless otherwise indicated, for every
kind of national protection available): AE, AG, AL, AM,

AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN,
CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI,
GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE,
KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD,
MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG,
PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ,
TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA,
ZM, ZW.

(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, GH,
GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM,
ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),
European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI,
FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO,
SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN,
GQ, GW, ML, MR, NE, SN, TD, TG).

Published:
— with international search report

(48) Date of publication of this corrected version:
9 September 2005

(15) Information about Correction:
see PCT Gazette No. 36/2005 of 9 September 2005, Sec-
tion II

For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.

(54) Title: **NON-HUMAN MAMMAL COMPRISING A MODIFIED SERCA2 GENE AND METHODS, CELLS, GENES, AND
VECTORS THEREOF**

(57) Abstract: The present invention relates to a non-human vertebrate, more particularly a non-human mammal, in which all cells contain genetic modifications in a Ca²⁺handling Serca ATPase gene, more particularly the Serca2 ATPase. Defective Ca²⁺ handling is induced in live animals by introducing genetic elements which direct the timing and specificity of the gene inactivation to the organ or tissue of interest; e.g. cardiac muscle cells. The primary application is to provide a standardized and reproducible animal model for heart failure or other human diseases, in which the defective Ca²⁺ handling function by the Serca gene can be manipulated in live animals. This is the only existing animal to date with such genetic modifications for this class of Ca²⁺handling proteins.

WO 2005/063007 A1